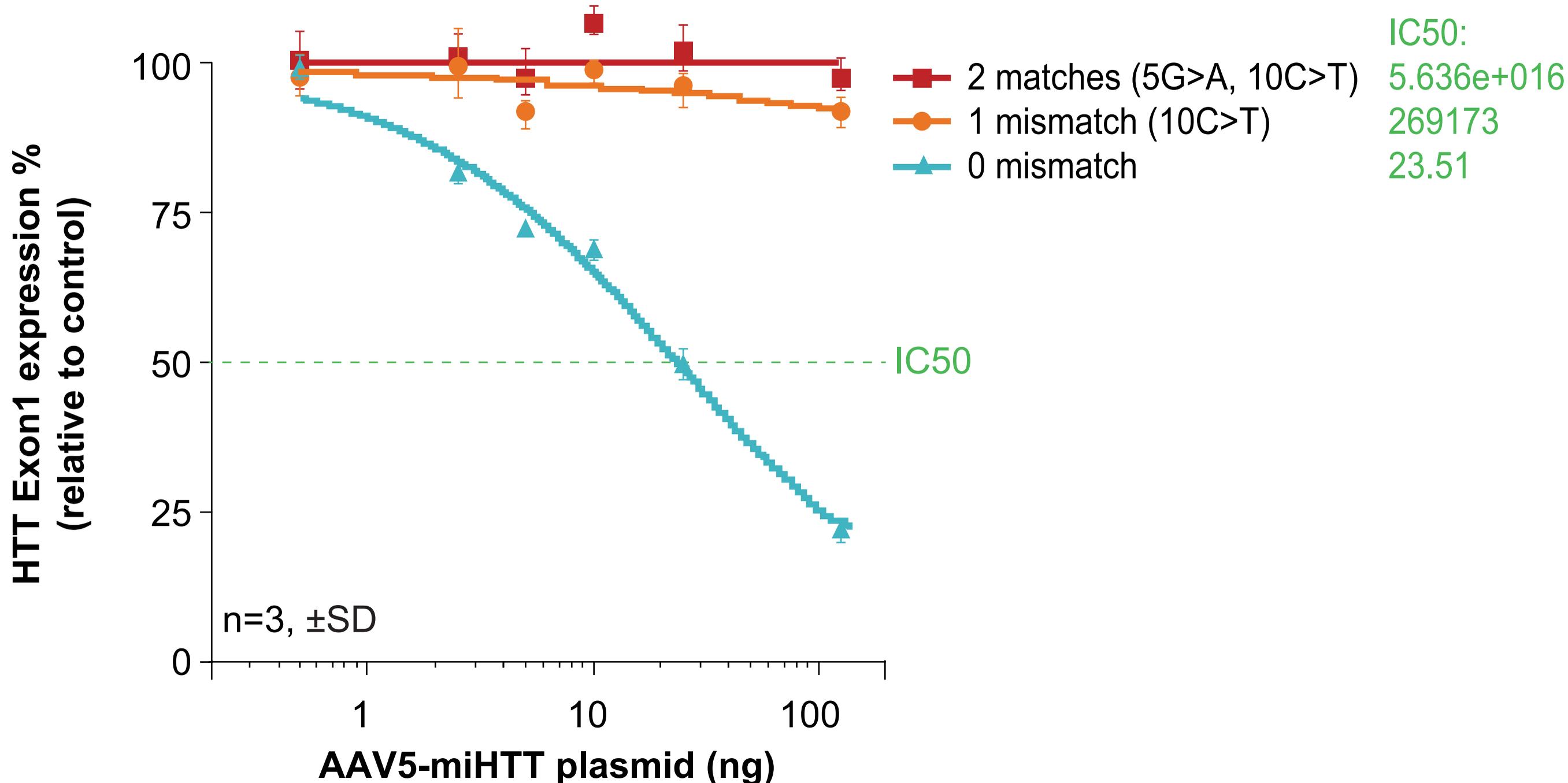


LUC HTT Exon1 reporter assay (25ng plasmid) in HEK293T cells



Supplementary figure 1. The Luciferase reporter assay showed a clear dose-dependent lowering of the luciferase reporter that has no mismatch to the AAV5-miHTT target sequence. The reporter with one mismatch, as is the case in the NHPs, did not show any lowering. Same holds true for the control reporter construct with 2 mismatches, confirming the validity of this assay.